

severe opportunistic infections, a major cause of transplant related mortality (Lang/ Handgretinger, BMT, 2008). We sought to determine engraftment, survival, IR, infection risk and frequency of PTL and GVHD in pediatric recipients following CD34-selected MUD PBSCT.

**Methods:** Pts  $\leq$  30 yrs requiring a MUD transplant for selective malignant and nonmalignant disease who were eligible. HLA matching included 8/10, 9/10 and 10/10 at intermediate resolution HLA A and B and high resolution HLA C, DRB1 and D2. Isolex 300i (Nexell, Irvine, CA) immunomagnetic cell selection system was used for CD34 selection with a goal of achieving  $\geq 5 \times 10^6$  CD34/kg PBSC. T cells were added back to reach a total CD3 dose of 1.0-2.6x10<sup>5</sup>/kg. GVHD prophylaxis consisted only of tacrolimus (Bhatia/Cairo et al, BBMT, 2010). Supportive care was as we have previously described (Bradley/Cairo, BMT, 2007).

**Results:** 19 pts, median f/u: 534d, median age: 15.3 yrs (10-23); 12:7 M:F, HLA match 26% 10/10, 31% 9/10, 42% 8/10; 68% malignant. Infused grafts contained a median of 1.6x10<sup>5</sup> CD3+/kg (0.1-4.8) and 5.1x10<sup>6</sup> CD34+/kg (2.0-13.3). Probabilities of neutrophil and platelet engraftment, grade II-IV aGVHD, cGVHD and day 100 TRM were 100%, 82.3%, 15.8%, 24.2% and 0%, respectively. CD3, CD4, CD8, CD19 and CD56 counts at day +180/365 reached normal in 11/30, 0/15, 22/46, 56/76 and 94/100% of patients, respectively. IgG, IgM, and IgA reached normal in 47/50, 59/50, and 59/33% of patients, respectively. One pt (5.2%) developed PTL. 11 donor/pts were CMV positive, and 2 pts experienced CMV SVI. The 1-yr probability of developing SVI and IFI was 42.3% and 28.0%, respectively. Despite the high incidence of SVI and IFI, the 1-yr probability of mortality due to SVI and IFI was 5.3% (CI95: 0-65%) and of OS was 84.2% (CI95: 59-95).

**Conclusions:** Rapid neutrophil engraftment, low rate of PTL, aGVHD/ cGVHD, and low day 100 TRM were observed. Although immune reconstitution was not mature at 1 yr post transplant, the overall probability of infection related mortality was relatively low. These results support continued investigation of CD34-selected MUD PBSCT in selective pediatric AlloSCT recipients.

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### HAPLOIDENTICAL STEM CELL TRANSPLANTATION FOR MINORITIES

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Hematopoietic stem cell transplants have been performed mostly from matched related or unrelated donors (MUD). Given the racial composition of the unrelated donor registries, it has been difficult to identify a MUD for non-Caucasian patients (pts). A review of all the MUD pts transplanted at MD Anderson (MDACC) past 25 years revealed that of 2117 pts, 1677 (79.2%) were whites (W), 271 pts (12.8%) Hispanics (H), 109 (5%) African-Americans (AA) and 33 (1.5%) were Asians (A). Similar racial distribution was noted with pts receiving a 9/10 MUD during the same period of time. We hypothesized that haploidentical stem cell transplantation (HaploSCT) would be an alternative for the minorities without a matched donor and treated 24 consecutive pts with a conditioning regimen consisting of fludarabine (40mg/m<sup>2</sup>/day  $\times$  4), melphalan (140mg/m<sup>2</sup>) and thiopeta (10mg/kg). 4 pts  $>$  55 years/comorbidities received a RIC with fludarabine and lower doses of melphalan (100mg/m<sup>2</sup>) and thiopeta (5mg/kg). GVHD prophylaxis consisted of post transplant cyclophosphamide (50mg/kg/day  $\times$  2), tacrolimus and mycophenolate.

**Results:** Racial distribution in this group was 8/24 (33.3%) W, 6/24 (25%) H, 5/24 (21%) AA, 4/24 (16.6%) A, 1/24 (4%) other, for a total of 66.6% minority population. Median age was 47 years (24-65). All pts but one received bone marrow stem cells. 4 pts had prior allogeneic transplants. 13 pts had AML/MDS (8 poor-risk cytogenetics), 6 pts had CML/MPD (5/5 blast phase CML), 5 pts had lymphoma/CLL. Donor-recipient HLA matching was: 5/10 in 12 pts (50%), 6/10 in 3/25 pts (12%), 7/10 in 5/25 pts (20%) and 8/10 in 4/25 (16%). 10 pts (42%) were in remission at the time of transplant. All 23 evaluable pts (one had early death) engrafted

with 100% donor chimerism (100% engraftment) after a median of 19 days (5-40). Cumulative incidence of day-100 treatment-related mortality (TRM) was 14%. No patients less than 50 years died of TRM. Grade II-IV aGVHD occurred in 4 patients and cGVHD in 1 patient. 7/18 (39%) pts relapsed while only 1 for the pts in remission at the time of transplant. After a median follow-up of 6 months (range 3-18) for survivors of  $>$  100 days (N = 14), OS for the whole group was 80%, while PFS for pts in remission at the time of transplant was 89% (N = 10, CI 43-98%). No differences in OS/PFS were noted between the Caucasian and non-Caucasian population.

**Conclusion:** HaploSCT is a feasible and safe transplant alternative for minority pts who lack a matched donor.

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### HLA-HAPLOIDENTICAL ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION USING DEPLETION OF CD3 $\pm$ CD19 IN CHILDREN AND ADOLESCENTS: EXPERIENCE AT A SINGLE INSTITUTION

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**Objective:** To evaluate the safety and efficacy of haploidentical HCT (h-HCT) with T-cell depleted graft in children and adolescents

**Patients and methods:** Between July 2008 and April 2010, 11 patients received allogeneic PBSCT from mismatched family donors. Of 11 patients, 6 had SAA (5 acquired, 1 Fanconi anemia), and 5 had hematologic malignancy (HM), of which 3 were AML (1 CR2, 1 refractory, 1 graft failure after CBT), 1 was ALL in CR3, and 1 was MDS-RA. A total of 13 h-HSCTs were performed in 11 patients. All 13 cases were conditioned with non-myeloablative regimen containing fludarabine, and busulfan at a dose of 8mg/kg was added for hematologic malignancies. ATG was also included except for cases of graft failure and none of enrolled patients received any kind of irradiation. The mobilized and collected product was processed for CD3 alone or CD3/CD19 depletion using CliniMACS®.

**Results:** Of 11 patients, 9 achieved neutrophil engraftment at a median of 11 days of whom 2 experienced late graft failure (GF) on day +25 and +200, respectively. Four patients developed acute GVHD of grade II and none had  $>$  II aGVHD. Of 4 patients who experienced GF (2 primary and 2 late), 2 received the 2nd h-HCT from different donors and all 2 are alive with complete donor chimerism. At a median follow-up of 12.2 (5.9~27.7) months, all 11 patients are alive. Of 6 patients with SAA, 4 are well and alive in a complete donor chimerism without transfusion (TF) need, 1 remains on infrequent need of TF with 95% donor chimerism at day +221, and 1 received a 2nd h-HCT for primary GF, after which the patient remains on infrequent need of TF with full donor chimerism at day +145. Of 5 patients with HM, 3 patients with AML are alive in CR at days +178, +412, and +812, respectively, although 1 of those experienced late graft failure. The remaining 2 patients are alive with disease at days +334 and +377, respectively, of whom 1 with MDS experienced 1° GF and the other with ALL relapsed at day +269. Although no infection-related death occurred, most of the patients had a viral reactivation or disease, including PTL and CMV retinitis.

**Conclusions:** The HCT using haploidentical family donors is a feasible option for children with diseases curable with HCT, but lack a suitable related or unrelated donor. However, graft failure and infection are still obstacles to overcome to improve the outcome of hHCT in children and adolescents.

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### TREOSULFAN-BASED CONDITIONING IS SUFFICIENT TO PROMOTE ENGRAFTMENT IN CORD BLOOD TRANSPLANTATION

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